

Friedreich's ataxia ▾

Specialty pharmacies: a guide for Friedreich's ataxia patients & carers

Understanding what specialty pharmacies are in the U.S. and why they are important for patients

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Introduction

Over 7,000 rare diseases affect over 30 million people in the United States [1]. A rare disease is defined as one that affects fewer than 200,000 people in the U.S. Navigating the healthcare system can be challenging for anyone, but it can be especially daunting for individuals and families dealing with a rare disease.

Recently, there have been many advancements in the diagnosis and treatment of rare diseases, likely due to financial incentives established by the Orphan Drug Act and scientific breakthroughs in genetics and precision medicine [2]. In 2023, over half of novel drug approvals by the FDA were to prevent, diagnose or treat rare diseases [3]. However, finding the right medications, understanding complex treatment plans, and managing costs are hurdles that rare disease patients face. Fortunately, specialty pharmacies offer services and support to help patients and their families on this journey.

This article will explore what specialty pharmacies are, how they work, and how they differ from traditional pharmacies. We will also examine recent trends and developments in this field and provide guidance on how patients can find the resources they need. We will provide examples of specialty pharmacies that specifically support Friedreich's ataxia patients.

This report is intended to empower patients with information but does not constitute an endorsement of any pharmacies listed.

What is a specialty pharmacy?

Specialty pharmacies focus on dispensing and managing medications for complex, chronic, or rare conditions that require an increased level of patient management [4]. Unlike traditional pharmacies, which primarily dispense common medications for more prevalent conditions, specialty pharmacies handle medications that often require specialized handling, storage, and administration such as injections or IV infusions [4] [5]. Whilst some pharmacies can be restricted to retail-only or mail-order-only pharmacies, specialty pharmacies may use either methods to deliver medications to you [4].

These pharmacies aim to offer comprehensive support, to ensure that every patient receives the attention and care they deserve, regardless of the complexity or rarity of their condition. Specialty pharmacies also help ensure proper usage and adherence [4].

How do specialty pharmacies work?

Specialty pharmacies operate differently from traditional pharmacies in several key ways. They often work closely with pharmaceutical manufacturers to manage limited distribution drugs (LDDs), which are specialty medications only available through a select number of pharmacies. This ensures that patients have access to these medications and receive the necessary support and monitoring. Here's a closer look at how specialty pharmacies work:

Dedicated teams: Specialty pharmacies have dedicated teams of pharmacists, nurses, and other healthcare professionals who are specially trained in rare diseases and their treatments. These teams provide personalized care, education, and support to patients and their families throughout their treatment journey [6].

For example, PANTHERx Rare Pharmacy utilizes a patient-centric model called RxARECARE, which involves dedicated care teams that provide compassionate support and streamline access to rare medications [7].

Comprehensive Services: Specialty pharmacies offer a wide range of services beyond simply dispensing medications to support patients. These services may include:

- **Benefits investigation and prior authorization support:** Helping patients navigate insurance coverage and obtain prior authorization for their medications [8].
- **Financial assistance programs:** Connecting patients with financial resources to help cover the costs of their medications [4].
- **Medication adherence support:** Providing reminders, education, and counseling to help patients take their medications as prescribed [8].

- **Side effect management:** Educating patients about potential side effects and providing support to manage them [4].
- **24/7 clinical support:** Offering round-the-clock access to pharmacists and nurses to answer questions and address concerns [9].

Specialized Infrastructure: Many rare disease medications require special handling, storage, and administration. Specialty pharmacies have the necessary infrastructure to meet these requirements, including:

- **Cold chain management:** Maintaining the appropriate temperature for medications that require refrigeration or freezing [9].
- **Specialized packaging and shipping:** Ensuring that medications are delivered safely and securely to patients' homes [9].
- **Medication administration:** Administering medication in forms such as injections or IV infusions [4].

Pharmacists in specialty pharmacies play a crucial role in guiding patients' journeys and monitoring pharmacotherapy [9]. They are actively involved in educating patients about their medications, assessing their response to treatment, and identifying potential risks or complications. This personalized approach ensures that patients receive the best possible care and achieve optimal outcomes.

How specialty pharmacies differ from traditional pharmacies

Specialty pharmacies differ significantly from traditional pharmacies in their focus, services, and expertise. Here's a table summarizing the key differences:

	Specialty Pharmacy	Traditional Pharmacy
Focus	Complex, chronic, or rare conditions	Common conditions
Medications	Specialty medications, often high-cost and requiring special handling	Commonly prescribed medications
Services	Comprehensive patient care, including benefits investigation, financial assistance, adherence support, and 24/7 clinical support	Primarily dispensing medications, with limited patient counseling
Staff	Specialized pharmacists and nurses with expertise in rare diseases	General pharmacists with broader knowledge

	Specialty Pharmacy	Traditional Pharmacy
Infrastructure	Specialized equipment for handling, storage, and administration of specialty medications	Standard pharmacy equipment
Distribution	Often limited to a select network of pharmacies	Widely available
Data Reporting	Systematic and customizable data reporting capabilities to meet manufacturer and payer requirements	Limited data reporting capabilities
Prior Authorization	Extensive prior authorization support and payment assistance	Limited prior authorization support
Accreditation	Can hold specialized accreditations, such as the ACHC Distinction in Rare Diseases and Orphan Drugs	May or may not hold specialty pharmacy accreditations

To achieve the ACHC Distinction in Rare Diseases and Orphan Drugs, specialty pharmacies must meet stringent standards, including specialized disease-specific training, development of disease management protocols, and capabilities for recording and reporting complex data [10].

Engaging with specialty pharmacies as a patient

Specialty medications, dispensed by specialty pharmacies, are not available over the counter and require a prescription from a healthcare provider first [4].

There are a number of factors that can dictate where a patient receives their medicine from, such as:

1. If an insurance plan has a designated in-network pharmacy a patient must use [4].
2. If the specialty medication is part of a limited distribution network, meaning it can only be dispensed by certain specialty pharmacies [4].

A patient usually engages with a specialty pharmacy **after** their clinician prescribes the medication and sends it to the appropriate pharmacy. At this point some pharmacies can help with insurance reviews & prior authorization support. Specialty pharmacies also provide education, financial assistance and care coordination for patients [5].

A patient may be able to select their specialty pharmacy if their insurance plan allows and the medication is available through multiple pharmacies.

How does a patient find which specialty pharmacies supply their medication?

Finding the right specialty pharmacy can be crucial for patients with rare diseases. Here are some ways patients can find specialty pharmacies that supply medication for their specific condition:

- **Consult with Your Healthcare Provider:** Your doctor or other healthcare provider is the best source of information about specialty pharmacies that specialize in your condition. They can provide referrals and recommendations based on your individual needs [2].
- **Contact the Pharmaceutical Manufacturer:** The manufacturer of your medication can often provide a list of specialty pharmacies that dispense their product. Many pharmaceutical companies have patient assistance programs that can help connect you with a specialty pharmacy and provide financial assistance [2].
- **Check with Your Insurance Company:** Your insurance company may have a preferred network of specialty pharmacies. Contact your insurer to find out which pharmacies they recommend.

Examples of specialty pharmacies providing Friedreich's ataxia treatments:

If you or a loved one want to explore a specialty pharmacy that provides treatments for Friedreich's ataxia, we have highlighted an example of a U.S.-based specialty pharmacy. Don't forget it often requires a prescription from your clinician first:

1. Biologics by McKesson

a. Website:

<https://biologics.mckesson.com/biologics-by-mckesson-named-the-exclusive-pharmacy-for-fda-approved-skyclarys-omaveloxolone-for-the-treatment-of-friedreichs-ataxia/>

b. Phone number: 800-850-4306

c. Email: mycareteam@biologicsinc.com

d. HQ based in Cary, North Carolina

Please note that Biologics by McKesson is the exclusive pharmacy for FDA-approved SKYCLARYS (omaveloxolone), a medication used for the treatment of Friedreich's ataxia. However, other pharmacies may also provide treatment for your condition. Some medications require specific administration methods, eligibility criteria, and financial approval. Patients should consult their physician, insurance provider, and the official manufacturer networks to confirm treatment eligibility, pharmacy access, and financial assistance availability. Always seek guidance from a licensed healthcare provider for treatment decisions.

Recent trends and developments

The field of specialty pharmacies is constantly evolving, with several recent trends and developments shaping the industry [9]:

- **Increased focus on rare and orphan diseases:** There has been a significant increase in drug development for rare and orphan diseases, driven by favorable legislation, scientific breakthroughs, and a growing recognition of the unmet needs of these patient populations [12]. This has led to a surge in the number of orphan drug approvals and a greater emphasis on personalized medicine [3].
- **Growth of gene therapy:** Gene therapy is an area of major growth in rare disease treatment, offering the potential to cure or significantly improve the lives of patients with genetic disorders [10]. However, gene therapy also presents challenges, such as its high cost and the need for specialized infrastructure and expertise.
- **Personalized medicine:** Advances in genetics and precision medicine are leading to more personalized treatments for rare diseases, with specialty pharmacies playing a key role in delivering these therapies and supporting patient care [2]. This trend is transforming the way rare diseases are diagnosed and treated, leading to more targeted and effective therapies.
- **Integrated health system specialty pharmacy (HSSP):** HSSPs offer a model of care that integrates specialty pharmacy services within health systems, providing a more seamless and coordinated experience for patients [11]. This model facilitates collaboration between healthcare providers, specialty pharmacies, and manufacturers, leading to better patient outcomes.

- **Technological advancements:** Technology is playing an increasingly important role in specialty pharmacies, with advancements in areas such as cold chain management, data reporting, and patient communication tools [9]. These advancements are improving efficiency, enhancing patient care, and supporting the delivery of complex therapies.
- **FDA's Rare Disease Innovation Hub:** The FDA has implemented a Rare Disease Innovation Hub to expedite the development and approval of safe and effective rare disease drugs [13]. This initiative aims to improve coordination between different agency centers and enhance support for rare disease drug development.
- **Orphan Drug Act (ODA):** The Orphan Drug Act (ODA) has been instrumental in increasing research into rare diseases [14]. It provides incentives for rare disease research and development, helping to bring new treatments to market.

While there have been significant advancements in rare disease drug development, challenges remain. These include the complex biology of many rare diseases, the small patient populations, and the high cost of treatments [15]. However, there are also significant opportunities for innovation and collaboration to address these challenges and improve the lives of patients with rare diseases.

Collaborations between health systems, specialty pharmacies, and manufacturers are crucial for facilitating advancements in rare disease treatment [11]. By working together, these stakeholders can improve patient access to care, optimize treatment strategies, and support the development of new therapies.



Conclusion

Specialty pharmacies can play a vital role in the lives of patients with rare diseases. They can provide access to life-saving medications, comprehensive support services, and specialized care that can significantly improve patients' quality of life. As the field continues to evolve, with a growing focus on personalized medicine, gene therapy, and technological advancements, specialty pharmacies are becoming increasingly important in ensuring that patients with rare diseases receive the best possible care and support.

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